

## Spinal Muscular Atrophy Program References

**Diverse small-molecule modulators of SMN expression found by high-throughput compound screening: early leads towards a therapeutic for spinal muscular atrophy.** Hum Mol Genet. 2005 Jul 15;14(14):2003-18.

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**Synthesis and Biological Evaluation of Novel 2,4-Diaminoquinazoline Derivatives as SMN2 Promoter Activators for the Potential Treatment of Spinal Muscular Atrophy.** J Med Chem. 2008 Feb 14;51(3):449-69.

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**DcpS as a Therapeutic Target for Spinal Muscular Atrophy.** ACS Chem Biol. 2008 Nov 21;3(11):711-22.

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**In Vivo Testing of D157495, a novel quinazoline compound, for the treatment of SMA.**

Rosalba Satta, Michael Jorgensen, Xue Gao, Jabir Singh, Jill Jarecki, Christine J. DiDonato.

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**National Institute of Neurological Disorders and Stroke Spinal Muscular Atrophy Fact Sheet**

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### Resources:

Families of Spinal Muscular Atrophy <http://www.fsma.org>

Muscular Dystrophy Association <http://www.mda.org>

National Institute of Neurological Disorders and Stroke <http://www.ninds.nih.gov>

Spinal Muscular Atrophy Foundation <http://www.smafoundation.org>